Media Release

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PHARMAXIS SUBMITS INVESTIGATIONAL NEW DRUG (IND) APPLICATION FOR THE TREATMENT OF MYELOFIBROSIS

Pharmaceutical research company Pharmaxis Ltd (ASX: PXS), the leading developer of therapeutics targeting lysyl oxidase enzymes to treat fibrosis and cancer, announced it has submitted an Investigational New Drug (IND) application to the US Food and Drug Administration (FDA) for a planned phase 1/2 study of PXS-5505 for the treatment of myelofibrosis. The protocol incorporates a one-month dose escalation phase followed by 6 months' treatment in an open label study of patients who are not on a JAK inhibitor. The study is planned to commence in Q4 2020 and expected to conclude in 2022.

"This IND submission incorporates the pre-IND feedback Pharmaxis received from the FDA earlier this year and is the next major step forward in the clinical development program of PXS-5505 for the treatment of myelofibrosis," said Mr Gary Phillips, Chief Executive Officer of Pharmaxis. "We are leveraging our leadership in lysyl oxidase science to bring new treatment options for these severely underserved patients and strongly believe that our novel approach of inhibiting all of the lysyl oxidase family members could reduce bone marrow fibrosis and have beneficial effects on blood cell production and consequently other aspects of the disease."

The IND application includes reports on the phase 1 studies in healthy volunteers that were completed in Q2 2020 as well as numerous individual studies that characterise the pharmacology, pharmacokinetics, and toxicology of PXS-5505 in a number of animal species, including the effects in various animal models of disease. Additionally, the application describes the GMP manufacture of the drug substance and drug product to be used in human clinical trials.

FDA feedback on the IND is expected within 30 days and Pharmaxis will outline the final study design and timing at that point.

Mr Phillips added, "After evaluating the safety and efficacy as a monotherapy in this first phase 1/2 study we plan further studies to include myelofibrosis patients being treated with JAK inhibitors which are the existing standard of care for many patients. We are also actively exploring how PXS-5505 can be progressed in a number of other fibrotic diseases and cancers, including pancreatic cancer, where we have compelling pre-clinical data."

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SOURCE: Pharmaxis Ltd, Sydney, Australia

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About Pharmaxis

Pharmaxis Limited is an Australian pharmaceutical research company and a global leader in drug development for inflammation and fibrotic diseases. The company has a highly productive drug discovery engine, drug candidates in clinical trials and significant future cash flows from partnering deals.

Leveraging its small-molecule expertise and proprietary amine oxidase chemistry platform, Pharmaxis has taken four in-house compounds to Phase 1 trials in just five years. Boehringer Ingelheim acquired the Pharmaxis anti-inflammatory AOC3 inhibitor in 2015 to develop it (BI 1467335) for two diseases: the liver condition Non-alcoholic Steatohepatitis (NASH) and diabetic retinopathy (DR).

The company's successor amine oxidase program has developed an oral anti-fibrotic LOXL2 inhibitor, aimed at NASH, pulmonary fibrosis (IPF) and other high-value fibrotic heart and kidney diseases, with a commercial partnering process underway, a systemic pan-LOX inhibitor for acute fibrosis and cancer that will enter a phase 2 study in 2020 and a topical pan-LOX inhibitor for scarring that is expected to commence phase 1 studies in 2H 2020. Pharmaxis' Mannitol platform has yielded the products Bronchitol® for cystic fibrosis, which is marketed in Europe, Russia and Australia, with United States FDA approval pending; and Aridol® for the assessment of asthma, which is sold in the United States, Europe, Australia and Asia.

Pharmaxis is listed on the Australian Securities Exchange (PXS). Its head office, manufacturing and research facilities are in Sydney, Australia. http://www.pharmaxis.com.au/

What is myelofibrosis?

Myelofibrosis is a disorder in which normal bone marrow tissue is gradually replaced with a fibrous scar-like material. Over time, this leads to progressive bone marrow failure. Under normal conditions, the bone marrow provides a fine network of fibres on which the stem cells can divide and grow. Specialised cells in the bone marrow known as fibroblasts make these fibres.
In myelofibrosis, chemicals released by high numbers of platelets and abnormal megakaryocytes (platelet forming cells) over-stimulate the fibroblasts. This results in the overgrowth of thick coarse fibres in the bone marrow, which gradually replace normal bone marrow tissue. Over time this destroys the normal bone marrow environment, preventing the production of adequate numbers of red cells, white cells and platelets. This results in anaemia, low platelet counts and the production of blood cells in areas outside the bone marrow for example in the spleen and liver, which become enlarged as a result.

Myelofibrosis can occur at any age but is usually diagnosed later in life, between the ages of 60 and 70 years. The cause of myelofibrosis remains largely unknown. It can be classified as either JAK2 mutation positive (having the JAK2 mutation) or negative (not having the JAK2 mutation).

Source: Australian Leukemia Foundation: https://www.leukaemia.org.au/disease-information/myeloproliferative-disorders/types-of-mpn/primary-myelofibrosis/

Forward-Looking Statements

Forward-looking statements in this media release include statements regarding our expectations, beliefs, hopes, goals, intentions, initiatives or strategies, including statements regarding the potential of products and drug candidates. All forward-looking statements included in this media release are based upon information available to us as of the date hereof. Actual results, performance or achievements could be significantly different from those expressed in, or implied by, these forward-looking statements. These forward-looking statements are not guarantees or predictions of future results, levels of performance, and involve known and unknown risks, uncertainties and other factors, many of which are beyond our control, and which may cause actual results to differ materially from those expressed in the statements contained in this document. For example, despite our efforts there is no certainty that we will be successful in partnering our LOXL2 program or any of the other products in our pipeline on commercially acceptable terms, in a timely fashion or at all. Except as required by law we undertake no obligation to update these forward-looking statements as a result of new information, future events or otherwise.